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Rare disease patients appeal to new Health Minister for urgent policy intervention

17 June 2024 | News

An immediate need to prioritise, ensure uninterrupted funding support for all notified ultra-rare diseases

With the third consecutive term win of the government, rare disease patients and their caregivers wrote to the newly appointed Union Minister at JP Nadda to draw Ministry's attention to the critical gaps that remain in the implementation of the National Policy for Rare Diseases (NPRD) 2021.

The lives of many patients (mostly children) suffering from chronic rare genetic disorders, such as Lysosomal Storage Disorders (LSDs), are still fraught with uncertainty due to the lack of sustainable funding and delays in the effective utilisation of the allocated funds.

The Centres of Excellence (CoEs), pivotal in this initiative, need to expedite the process to ensure timely access to life-saving treatments.

There is an immediate need to prioritise, ensure uninterrupted funding support for all notified ultra-rare diseases, conditions which are chronic in nature, but have approved therapies with excellent clinical outcomes. The current budgetary provision up to Rs 50 lakh is only one-time, thereby hindering the process of saving lives for patients diagnosed with these conditions. Several patients across the Centres of Excellence (CoEs) have once again been put off life-saving therapies after the one-time support exhausted.

Although notified as a Group 3(a) condition in the NPRD 2021, patients diagnosed with Pompe disease, Fabry disease, MPS I and II are still not getting the same priority at the CoEs, thereby delaying treatment and posing serious risk to their lives.

Many of the CoEs have still not been able to effectively utilise the funds provided by the Ministry, thereby limiting access to life-saving therapy. Repeated appeals to the CoEs and the Ministry in this regard have not yielded any impact so far.